Clinical Development of a Cell Therapy for Diabetes

Grant Award Details

Clinical Development of a Cell Therapy for Diabetes

Grant Type: Accelerated Development Pathway I

Grant Number: AP1-08039

Project Objective: Complete Step 1 Phase I/II Clinical Trial using VC-01-250 devices and launch Phase II Bridging Study using EN-LC device

Investigator:

Name:	Howard Foyt
Institution:	ViaCyte, Inc.
Type:	PI

Disease Focus: Diabetes, Type 1 diabetes

Human Stem Cell Use: Embryonic Stem Cell

Cell Line Generation: Embryonic Stem Cell

Award Value: $8,783,852

Status: Active

Grant Application Details

Application Title: Clinical Development of a Cell Therapy for Diabetes

Public Abstract:

We are developing a stem cell-derived replacement cell therapy for insulin-requiring diabetes. Through a process known as directed differentiation, embryonic stem cells are turned into pancreatic cells in the laboratory. The pancreatic cells are loaded into a delivery device, which is essentially a small envelope made with a semi-permeable membrane, not unlike a flat tea bag. When the cells in the device (combination product) are implanted under the skin, they become pancreatic endocrine cells, including insulin-producing beta cells that respond to elevated blood glucose by releasing insulin in a physiologic manner. The prototype combination product has been tested in hundreds of animals, is routinely curative in a mouse model of chemically-induced diabetes, and has been shown to be safe in several animal studies. Moreover, the delivery device has been shown to protect cells from a recipient's immune system. The Team has received valuable feedback from the FDA, and we plan to launch the first clinical test of our therapeutic candidate in patients with diabetes in 2014. This first clinical trial will utilize the prototype to establish safety in humans, and determine the dosing range that might provide benefit to patients with diabetes.

The current application is to fund additional clinical research, and associated product development activity, that will (1) ensure the first trial is executed in a most informative and timely fashion, (2) accelerate the pace at which information is collected on how the product works in humans – testing various formats, and in different types of patients – and (3) substantially increase the likelihood that the most appropriate format and patient population is selected for a definitive "Phase 3" clinical trial. A Phase 3 trial serves as the basis for an application to the FDA to obtain a license to market the product. In this way, CIRM Accelerated Development Pathway designation of the project will substantially increase the probability that, and pace at which, this product concept becomes a real treatment available to the millions of patients in need.

Statement of Benefit to California:

Diabetes mellitus currently afflicts approximately 370 million people worldwide, with projections of over 550 million by the year 2030 (sources: World Health Organization; International Diabetes Federation). In the year 2000 there were approximately 2 million cases of diabetes in California (source: Diabetes Control Program, California Department of Health Services). Further, the disease disproportionately affects certain minority groups and the elderly. Despite the use of insulin and advances in its delivery, the human cost of diabetes is underscored by the financial costs to society: tens of billions of dollars each year in California alone. The primary cause of type 1 diabetes, and contributing significantly to type 2 diabetes as well, is the loss of insulin-producing pancreatic beta cells. The CIRM Diabetes Disease Team Project is developing an innovative beta cell replacement therapy for insulin-requiring diabetes. If successful, the therapy will go beyond insulin function, and will perform the full array of normal beta cell functions, including responding in a more physiological manner than manual or mechanized insulin self-administration. Because they will be more physiological, the replacement cells could reduce the long-term effects of diabetes. Moreover, the cell therapy will alleviate patients of the constant monitoring of blood glucose, painful insulin injections, and the ever-present risk of overdosing with insulin. For these reasons, it is possible that the product could transform the diabetes treatment landscape dramatically and even replace pharmaceutical insulin in the market. This product will be available in California first, through clinical testing, and if approved by the FDA for commercial production, will eventually help hundreds of thousands of Californians with diabetes. The product will substantially increase quality of life for patients and their families, while significantly reducing the health care burden in the state. The proposed project will employ Californian doctors and scientists, and success will prove highly noteworthy for the state. Lastly, once commercially marketed, the product will yield additional jobs in manufacturing, sales, and related industries, and generate revenue for California. Given the market need and the clear feasibility, the product could become the most significant stem cell-based medical treatment of the coming decade, and that will be a tremendous achievement for California, its taxpayers, and CIRM.